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(Original Signature of Member)

114TH CONGRESS  
1ST SESSION

# H. R.

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To amend the Federal Food, Drug, and Cosmetic Act with respect to expanding access for breakthrough drugs, and for other purposes.

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## IN THE HOUSE OF REPRESENTATIVES

Mr. MCCAUL introduced the following bill; which was referred to the Committee on \_\_\_\_\_

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# A BILL

To amend the Federal Food, Drug, and Cosmetic Act with respect to expanding access for breakthrough drugs, and for other purposes.

1 *Be it enacted by the Senate and House of Representa-*  
2 *tives of the United States of America in Congress assembled,*

3 **SECTION 1. SHORT TITLE.**

4 This Act may be cited as the “Andrea Sloan Compas-  
5 sionate Use Reform and Enhancement Act” or the “An-  
6 drea Sloan CURE Act”.

1 **SEC. 2. EXPANDED ACCESS POLICY AS CONDITION OF EX-**  
2 **PEDITED APPROVAL.**

3 Section 561 of the Federal Food, Drug, and Cosmetic  
4 Act (21 U.S.C. 360bbb) is amended—

5 (1) by redesignating subsections (d) and (e) as  
6 subsections (e) and (f), respectively; and

7 (2) by inserting after subsection (c) the fol-  
8 lowing new subsection:

9 “(d) EXPANDED ACCESS POLICY REQUIRED FOR  
10 COVERED INVESTIGATIONAL DRUGS.—

11 “(1) IN GENERAL.—With respect to a covered  
12 investigational drug, not later than 30 days after the  
13 date on which the drug meets the definition of a cov-  
14 ered investigational drug (as specified in paragraph  
15 (2)), the sponsor of the covered investigational drug  
16 shall submit to the Secretary, and make publicly  
17 available, the policy of the sponsor with respect to  
18 requests submitted under subsection (b). In the case  
19 of such a policy under which the sponsor accepts  
20 such requests, such policy shall include—

21 “(A) a single point of contact who receives  
22 and processes such requests;

23 “(B) procedures for making such requests;

24 “(C) the general criteria for the sponsor’s  
25 consideration or approval of such requests; and

1           “(D) the amount of time the sponsor an-  
2           ticipates will be necessary to respond to such  
3           requests.

4           “(2) COVERED INVESTIGATIONAL DRUG.—In  
5           this subsection, the term ‘covered investigational  
6           drug’ means a drug that—

7                   “(A) is designated as a breakthrough ther-  
8                   apy or as a fast track product;

9                   “(B) is designated under section 505E(d)  
10                  as a qualified infectious disease product; or

11                  “(C) is designated under section 526 as a  
12                  drug for a rare disease or condition.”.

13   **SEC. 3. NOTIFICATION OF SUBMITTERS OF EXPANDED AC-**  
14                   **CESS REQUESTS.**

15           Section 561 of the Federal Food, Drug, and Cosmetic  
16   Act (21 U.S.C. 360bbb), as amended by section 2, is fur-  
17   ther amended—

18                   (1) by redesignating subsections (e) and (f) (as  
19                   redesignated by section 2(1)) as subsections (f) and  
20                   (g), respectively; and

21                   (2) by inserting after subsection (d) (as in-  
22                   serted by section 2(2)) the following new subsection:

23           “(e) NOTIFICATION OF SUBMITTERS OF RE-  
24   QUESTS.—In the case of the denial by a manufacturer or  
25   distributor of a request under subsection (b), not later

1 than 5 days after the date of such denial, the manufac-  
2 turer or distributor, as applicable, shall submit to the per-  
3 son (or physician) who made the request written notice  
4 of the denial, including an explanation for the denial.”.

5 **SEC. 4. GAO QUALITATIVE ANALYSIS ON INDIVIDUAL PA-**  
6 **TIENT ACCESS TO UNAPPROVED THERAPIES**  
7 **AND DIAGNOSTICS.**

8 Not later than 180 days after the date of the enact-  
9 ment of this Act and every two years thereafter through  
10 2023, the Comptroller General of the United States shall  
11 submit to the Committee on Energy and Commerce of the  
12 House of Representatives and the Committee on Health,  
13 Education, Labor and Pensions of the Senate a report  
14 containing a qualitative analysis of the extent to which in-  
15 dividual patients have access to investigational drugs pur-  
16 suant to subsection (b) of section 561 of the Federal Food,  
17 Drug, and Cosmetic Act (21 U.S.C. 360bbb) and rec-  
18 ommendations for improving such access. In preparing  
19 such report, the Comptroller General shall conduct a qual-  
20 itative analysis of the following:

21 (1) Whether there are any identifiable patterns  
22 in requests submitted under subsection (b) of such  
23 section, such as the types of indications for which  
24 requests for individual patient access are sought or  
25 the reasons for the denial of such requests.

1           (2) What the primary barriers are to drug  
2 sponsors granting requests for individual patient ac-  
3 cess.

4           (3) How the Secretary evaluates safety and effi-  
5 cacy data submitted in connection with such re-  
6 quests.

7           (4) The amount of time that—

8                 (A) a physician typically takes to complete  
9 the paperwork necessary to make such a re-  
10 quest;

11                (B) a drug sponsor takes to process such  
12 a request and to issue a decision with respect  
13 to the request; and

14                (C) the Secretary takes to process such a  
15 request and to issue a decision with respect to  
16 the request.

17           (5) How regulations, guidance, policies, or prac-  
18 tices may be modified, streamlined, expanded, or dis-  
19 continued to reduce or prevent delays in approving  
20 such requests.

21           (6) The number of such requests that, for the  
22 period covered by the report—

23                 (A) were approved by drug sponsors and  
24 the Food and Drug Administration;

1 (B) were approved by drug sponsors but  
2 denied by the Food and Drug Administration;  
3 and

4 (C) were denied by drug sponsors.

5 (7) How to encourage drug sponsors to grant  
6 requests for expanded access under such section  
7 561, including requests for emergency use, inter-  
8 mediate-size patient populations, and large patient  
9 populations under a specified indication.

10 (8) Whether and to what extent adverse events  
11 reported to the Secretary as a result of individual  
12 use of an investigational drug or investigational de-  
13 vice under such section 561 affected the development  
14 or approval of any drug or device.

15 **SEC. 5. EXPANDED ACCESS TASK FORCE.**

16 (a) ESTABLISHMENT.—The Secretary of Health and  
17 Human Services shall establish a task force within the De-  
18 partment of Health and Human Services to explore mech-  
19 anisms for improving the access individual patients have  
20 to investigational drugs pursuant to subsection (b) of sec-  
21 tion 561 of the Federal Food, Drug, and Cosmetic Act  
22 (21 U.S.C. 360bbb), to be known as the “Expanded Ac-  
23 cess Task Force” (in this section referred to as the “Task  
24 Force”). Not later than 90 days after the date on which  
25 the Comptroller General of the United States submits the

1 first report required under section 4, the Task Force shall  
2 be convened.

3 (b) MEMBERSHIP.—

4 (1) COMPOSITION.—The Task Force shall be  
5 composed of not more than 13 voting members ap-  
6 pointed as follows:

7 (A) One member to serve as Chairman of  
8 the Task Force, appointed by the Speaker of  
9 the House of Representatives.

10 (B) One representative from the Depart-  
11 ment of Health and Human Services, appointed  
12 by the Secretary of Health and Human Serv-  
13 ices.

14 (C) Six representatives appointed by the  
15 majority leader of the House of Representa-  
16 tives, in consultation with the minority leader of  
17 the House of Representatives, and the chairman  
18 and the ranking member of the Committee on  
19 Energy and Commerce of the House of Rep-  
20 resentatives, including—

21 (i) one current or former representa-  
22 tive of the biopharmaceutical industry of  
23 not less than 250 full-time employees;

1 (ii) one representative of a biopharma-  
2 ceutical company of less than 250 full-time  
3 employees;

4 (iii) one representative of the patient  
5 community;

6 (iv) one representative of the rare dis-  
7 ease patient community;

8 (v) one representative of the health  
9 care provider community; and

10 (vi) one bioethicist.

11 (D) Five representatives appointed by ma-  
12 jority leader of the Senate, in consultation with  
13 the minority leader of the Senate, and the  
14 chairman and the ranking member of the Com-  
15 mittee on Health, Education, Labor and Pen-  
16 sions of the Senate, including—

17 (i) one representative of the bio-  
18 pharmaceutical industry of not less than  
19 250 full-time employees;

20 (ii) one current or former representa-  
21 tive of a biopharmaceutical company of  
22 less than 250 full-time employees;

23 (iii) one representative of the patient  
24 community;

1 (iv) one representative of the rare dis-  
2 ease patient community; and

3 (v) one representative of the health  
4 care payor community.

5 (2) COMPENSATION.—Members of the Task  
6 Force shall serve without compensation.

7 (c) DUTIES.—The Task Force shall comprehensively  
8 evaluate the access individual patients have to investiga-  
9 tional drugs pursuant to subsection (b) of section 561 of  
10 the Federal Food, Drug, and Cosmetic Act (21 U.S.C.  
11 360bbb), taking into account—

12 (1) the unique challenges faced by children with  
13 likely fatal diseases for which there is not a com-  
14 parable or satisfactory alternative therapy available;

15 (2) possible incentives for biopharmaceutical  
16 companies and providers to approve requests sub-  
17 mitted under such subsection;

18 (3) ways to improve followup reporting of ad-  
19 verse event data and compliance with such reporting  
20 requirements;

21 (4) how the Secretary of Health and Human  
22 Services interprets and takes into consideration ad-  
23 verse event data reported in the case of data from  
24 use under a request submitted under such sub-  
25 section;



1 lished under section 5 submits the report under subsection  
2 (d) of such section, the Secretary of Health and Human  
3 Services shall finalize the draft guidance entitled “Ex-  
4 panded Access to Investigational Drugs for Treatment  
5 Use—Qs & As” and dated May 2013.

6 (b) CONTENTS.—The final guidance referred to in  
7 subsection (a) shall—

8 (1) clearly define how the Secretary interprets  
9 and uses adverse drug event data reported by inves-  
10 tigators in the case of data reported from use under  
11 a request submitted under section 561(b) of the  
12 Federal Food, Drug, and Cosmetic Act (21 U.S.C.  
13 360bbb(b)); and

14 (2) take into account the report of the Ex-  
15 panded Access Task Force submitted under section  
16 5(d) and the first report of the Comptroller General  
17 of the United States submitted under section 4.